

## REMARKS

Claims 1-29 are presently pending in the instant Application. In the instant Amendment, Applicants have canceled Claims 8, 13, 21, 24 and 29, have amended Claims 1-7, 9-12, 14-20, 22-23, and 26-28, and have added new Claims 30-36. Support for amended Claims 1-7, 9-12, 14-20, 22-23, and 26-28, as well as for new Claims 30-36 can be found generally throughout the instant Specification, and particularly at page 74, lines 17-28; pages 75-77; Figures 8-10, and Claims 1-28 as originally filed.

### *Drawings*

The Examiner has acknowledged that drawings filed as part of the instant Application are adequate for purposes of examination. The Applicants are grateful the Examiner's acknowledgement.

### *Priority*

The Examiner has asserted that Applicants have not complied with one or more conditions for receiving the benefit of an earlier filing date under 35 U.S.C. 119(e). In particular, the Examiner believes that an application in which the benefits of an earlier application are desired (i.e. 60/085,845, and PCT/FR99/00740) must contain a specific reference to the prior application(s) in the first sentence of the specification or in an application data sheet (37 CFR 1.78(a)(2) and (a)(5)). The specific reference to any prior nonprovisional application must include the relationship (i.e., continuation, divisional, or continuation-in-part) between the applications except when the reference is to a prior application of a CPA assigned the same application number.

In the instant matter, the Examiner has acknowledged receipt of a certified copy of the foreign application referred to in the oath or declaration or in an application data sheet. The Examiner has asserted that if the instant Application has entered the national stage from an

international application filed on or after November 29, 2000, after compliance with 35 U.S.C. § 371, the claim for priority must be made during the pendency of the instant Application and within the time limit set forth in the PCT and regulations of the PCT.

In response, Applicants respectfully submit a priority claim was made timely in the instant Application. Indeed, an original filing receipt mailed February 5, 2001 acknowledges that the instant Application is a 371 of PCT/FR99/00740 filed March 30, 1999, and claims priority from French Application 98/04121 filed April 2, 1998. Moreover, an updated filing receipt mailed July 9, 2002 confirms all of the information set forth in the original filing receipt, and further acknowledges that PCT/FR99/00740 claims the benefit of U.S. Provisional Application 60/085,845 filed May 18, 1998.

It is respectfully submitted the instant Amendment to the Specification adding the Priority Claim is indeed timely and should be allowed. MPEP §201.14(a) clearly states:

For applications that entered the national stage from an international application filed ***on or after*** November 29, 2000, after compliance with 35 U.S.C. § 371, the claim for priority must be made during the pendency of the application and within the time limit set forth in the PCT and Regulations under the PCT. Any foreign priority claim not presented within the time period set forth in 37 CFR 1.55(a)(1)(i) is considered to have been waived.

(MPEP § 201.14(a) (emphasis added)).

As the Examiner is well aware, PCT Application PCT/FR99/00740 was filed on March 30, 1999, which is ***prior*** to November 29, 2000. Hence, the time period set forth in 37 CFR 1.55(a)(1)(i) is not applicable in this case, and the amendment of the instant Specification to include a Priority Claim at this time is timely and appropriate.

***The Claims are Acceptable***

The Examiner has objected to Claim 1. Initially, the Examiner believes the word “bond” should replace the word “bonding” recited in Claim 1 because, in the Examiner’s opinion, an

object (“R<sub>2</sub>”) is specified and not an action. The Examiner also has asserted that, alternatively, the words “site for” could be inserted directly after “bonding”. The Examiner also believes a similar situation exists at page 80, lines 12-18. Also with respect to Claim 1, the Examiner has asserted the word “The” recited at page 80, line 7 should not be capitalized, and that the asterisks at page 80, lines 8 and 14, and at page 82, lines 10, 17, and 18 should be replaced with bullets or some other character, because the Examiner is of the opinion that asterisks were defined in Claim 1 at page 79, lines 20-22 as a hydrogen atom or a bonding site.

Furthermore, the Examiner has objected to Claim 8 because, in the Examiner’s opinion, the term “chotestanyl” at line 19 is misspelled.

The Examiner has also objected to Claims 10-12 because he believes they are incomplete sentences inasmuch as they lack an article modifying the noun “Method”. The Examiner has suggested the word “A” be inserted.

Moreover, the Examiner has objected to Claim 18 for lacking a comma after “histone”, and has objected to Claims 18-21 under 37 CFR § 1.75(c) as being in improper form because a multiple dependent claim cannot depend from another multiple dependent claim. It is the Examiner’s position that Claims 19-21 depend from Claims 12-19, of which Claim 14 is a multiple dependent claim. Hence, the Examiner has not considered Claims 19-21 on the merits.

In response, it is respectfully submitted that the instant Claims as amended herein are in acceptable form. In particular, Claim 1 has been amended to recite, *inter alia*, “...X represents a group NR<sub>2</sub> or CHR<sub>2</sub>, R<sub>2</sub> being either a hydrogen atom or the **bonding site** for R<sub>1</sub> (emphasis added)”. This correction has also been made at page 80, lines 12-18. Moreover, the Claim has been amended so that the word “The” recited on page 80, line 7 is no longer recited, and that asterisks at page 80, lines 8 and 14, and at page 82, lines 10, 17, and 18 have been replaced with bullets.

Furthermore, Claim 8 has been amended to recite the term “cholestanyl” rather than “chotestanyl”, Claims 10-11 have been amended to be directed towards, *inter alia*, “A method...”, Claim 12 has been amended to be directed towards, *inter alia*, “A composition...”, Claim 18 has been amended to have a comma after “histone”, Claims 19-20 have been amended to no longer be multiple dependent Claims, and Claim 21 has been canceled, without prejudice.

For all of the foregoing reasons, it is respectfully submitted that Claims 1, 8, 10-12 and 18-20 are in correct form, and these objections should be removed.

***The Claims are Drawn to Patentable Subject Matter***

Claims 24 and 25 have been rejected under 35 U.S.C. § 101 because the claimed recitation of use, without setting forth any steps involved in the process, results in an improper definition of a process, i.e., results in a claim which is not a proper process under 35 U.S.C. § 101.

Initially, Applicants respectfully point out to the Examiner that Claim 25 was deleted in a preliminary amendment filed 21 November 2000 with the USPTO. With respect to Claim 24, in the instant Amendment, Claim 24 has been canceled, without prejudice. Hence, this rejection is MOOT.

***The Invention is Definite***

Claims 1-29 have been rejected under 35 U.S.C. § 112, second paragraph as being indefinite for failing to particularly point out and distinctly claim the subject matter regarded to be the invention. The Examiner has assert that, although Claims 12, 26 and 28 are independent Claims, they require a “compound of general formula I”, which is defined in Claim 1. Hence, the Examiner believes many of the indefiniteness rejections that apply to Claim 1 also apply to claims 12, 265, and 28, as well as to Claims dependent upon these Claims.

Firstly, the Examiner has asserted that Claims 1-25, and 29 are indefinite because they are drawn to a compound in a “DL form”. The Examiner believes such a form is not possible because, in the Examiner’s opinion, a pure solution of a compound either rotates plane polarized light to the right (D), or to the left (L), and that a single compound can not in the end do both. It is the Examiner’s opinion that the term DL (or D,L) is used to refer to racemic mixtures of D compounds and L compounds.

In the instant Amendment, Claim 1 has been amended to be drawn to, *inter alia*, “A compound, a stereoisomer of said compound, a salt of said compound, or a salt of said stereoisomer....” As the Examiner is well aware, D and L isomers of a compound are stereoisomers, and at page 3, lines 21-24 of the instant Application, as well as in Claim 1 as filed, Applicants discuss D and L isomers of a compound of the instant Invention. Hence, amended Claim 1 clearly does not introduce new matter into the instant Application.

Furthermore, the Examiner believes that Claims 1-8, 10-25, and 29 are definite because they recite “the different groups  $[(CH_2)_p-Y]$ ” without proper antecedent basis. The Examiner has asserted that this phrase implies that there must be different  $[(CH_2)_p-Y]$  groups. Yet, the Examiner is the opinion that, according to the Claims, as few as 0 or 1  $[(CH_2)_p-Y]$  groups are allowed. Thus, the Examiner believes there need not be different  $[(CH_2)_p-Y]$  groups. The Examiner has also asserted that a similar situation arises at page 81, lines 7-8, lines 9-10, and lines 16-17 with respect to “the different groups  $NR_4-(CH)_r-$ , “the different groups  $-NR_4-(CH)_rR_3$ ”, and “the different groups  $-(CH_2)_s-NR_5$ ”.

In the instant Amendment, claim 1 has been amended to be directed towards, *inter alia*, a compound in which “...Y represents a carbonyl, amino, methylamino, or methylene group, wherein Y need not be identical in each  $[(CH_2)_p-Y]$  group **should  $2 \leq q \leq 10$ ...**(emphasis added).” Thus, this Amendment makes it readily clear that in a situation where there are more than one

$[(\text{CH}_2)_p\text{-Y}]$  group, i.e. if  $q$  of general formula (III) is greater than or equal to 2 and less than or equal to 10,  $Y$  need not be the same in each  $[(\text{CH}_2)_p\text{-Y}]$  group. Claim 1 has also been amended to state *inter alia*, that:

(a) “... $r$  is an integer between 0 and 10 inclusive, wherein  $r$  need not have the same value in each  $-\text{NR}_4\text{-}(\text{CH})_r-$  group should  $2 \leq t \leq 8...$ ” should  $t$  of general formula (VI) be greater than or equal to 2 and less than or equal to 8, and

(b) “... $R_3$  represents a hydrogen atom, a methyl group or a group of general formula (VII):...for which  $u$  is an integer between 1 and 10 inclusive,  $s$  is an integer between 2 and 8 inclusive *and need not have the same value in each  $-(\text{CH}_2)_s\text{-NR}_5$  group of general formula (VII) should  $2 \leq q \leq 10$* ,  $R_5$  is a hydrogen atom or a CA group as defined above, *wherein the CA group in each  $-(\text{CH}_2)_s\text{-NR}_5$  group of general formula (VII) need not be identical should  $2 \leq u \leq 10$ , and wherein  $R_3$  need not be identical in each  $\text{NR}_4\text{-}(\text{CH})_rR_3$  group of general formula (VI) should  $2 \leq t \leq 8...$* (emphasis added).”

It is respectfully submitted that these amendments readily make clear to one of ordinary skill in the art that in a compound of the instant Invention wherein there is more than one  $-\text{NR}_4\text{-}(\text{CH})_r-$  group, more than one  $-(\text{CH}_2)_s\text{-NR}_5$  group, more than one  $-(\text{CH}_2)_s\text{-NR}_5$  containing a CA group, and/or more than one  $\text{Nr}_4\text{-}(\text{CH})_rR_3$  group, they need not be identical. The various compounds of the instant Invention as set forth in the Specification and Claims readily provide support for these amendments.

In addition, the Examiner has asserted that Claims 1-8, 10-25, and 29 are indefinite because, in the Examiner’s opinion, the metes and bounds of “ $Y$ ” are unclear. The Examiner believes the Claims limit ‘ $Y$ ’ to one of carbonyl, amino, methylamino, and methylene, but then indicates that it may have “different meanings within the same groups.” In the Examiner’s opinion, this phrase could mean that ‘ $Y$ ’ is limited to one of the indicated entities, or that ‘ $Y$ ’

could have some other disclosed identity. For similar reasons, the Examiner also believes that the metes and bounds of “r” are unclear as defined at page 81, lines 6-8, because the Examiner believes that the Claims limit ‘r’ on the one hand to be between 0 and 10 inclusive, but subsequently allow ‘r’ to have “different meanings.” The Examiner believes a similar situation exists for the term ‘s’ at page 81, lines 14-16, the term ‘CA’ at page 81, lines 18-19 and page 82, lines 4-6, and for general formula (VII) at page 82, lines 1 and 2.

In response, it is respectfully submitted that in the instant Amendment, amended Claim 1 states that Y represents a carbonyl, amino, methylamino or methylene group, but that in each  $[(CH)_p-Y]$  group, *should q of general formula (III) be greater than or equal to 2 and less than or equal to 10*, Y does not need to be identical in each  $[(CH)_p-Y]$  group. A similar amendment has been made for “r”, i.e. that r is an integer between 0 and 10 inclusive, but that r need not have the same value in each  $-NR_4-(CH_r)-$  group should there be more than one  $-NR_4-(CH_r)-$  group in the compound. Thus, amended Claim 1 makes clear that r can have a value of 0, 1, 2, 3, 4, 5, 6, 7, 8, 9, or 10, but that value can be different in each  $-NR_4-(CH_r)-$  group in the compound, should the compound contain more than one  $-NR_4-(CH_r)-$  group

It is also the Examiner’s opinion that Claims 1-8, 10-25, and 29 are indefinite because they lack proper antecedent basis for the phrase “whose nitrogen atom” at page 81, line 2 in the description of general formula (VI). The Examiner has asserted that general formula (VI) allows for up to 8 nitrogens. Thus, the Examiner believes insertion of the word “terminal” should be inserted immediately before “nitrogen”.

In the instant Amendment, Claim 1 has been amended to state that “Rep is absent or is a spacer of general formula (VI), wherein the *terminal* nitrogen atom is attached to atoms X, V, W, or Z of general formula (II) when the compound comprises general formula (IV)...(emphasis added).

Moreover, the Examiner has asserted that Claims 1-8, 10-25, and 29 are indefinite because the phrase “depending on the cases” is confusing. In the Examiner’s opinion, the only antecedent for “the cases” is the recitation of the “1<sup>st</sup> case” and “2<sup>nd</sup> case”, but the Examiner believes the Claim does not clearly indicate that these are what is referred to, and the nature of recited dependency is unclear. In order to overcome this rejection, the Examiner believes the Claim should be amended to state “whose terminal nitrogen is attached to one of atoms X, V, W, or Z of general formula (II) when the compound comprises general structural formula (IV), or to the substituent Y of the group R<sub>1</sub> when the compound comprises general structural formula V.”

In the instant Amendment, Claim 1 has been amended so that it no longer recites the phrases “1<sup>st</sup> case”, “2<sup>nd</sup> case”, and “the cases”. Rather, as explained above, amended Claim 1 states “...wherein the terminal nitrogen is attached to atoms X, V, W or Z of general formula (II) when the compound comprises general formula (IV), or to the substituent Y of the group R<sub>1</sub> when the compound comprises general formula (V)....”

In addition, the Examiner has asserted that Claims 1-8, 10-23 and 29 are indefinite because the metes and bounds of “steroid derivative” is unclear. It is the Examiner’s position that neither the instant Specification nor the Claims defines this term. Hence, the Examiner believes one of skill in the art cannot know the metes and bounds of the Claims.

In response, it is respectfully submitted that Claim 1 has been amended to define a “steroid derivative” to be “...selected from the group consisting of cholesterol, cholestanol, 3- $\alpha$ -5-cyclo-5- $\alpha$ -cholestan-6- $\beta$ -ol, cholic acid, cholesteryl formate, cholestanyl formate, 3 $\alpha$ ,5-cyclo-5 $\alpha$ -cholestan-6 $\beta$ -yl formate, cholesterylamine, 6-(1,5-dimethylhexyl)-3a,5a-dimethylhexadecahydrocyclopenta[a]cyclopropa[2,3]cyclopenta[1,2-f]naphthalen-10-ylamine, and cholestanylamine....” This definition was set forth in Claim 8 as originally

filed. Hence, contrary to the Examiner's assertions, a "steroid derivative" is clearly defined in the instant Application, and this amendment introduces no new matter.

The Examiner further contends that Claims 1-8, 10-25 and 29 are indefinite because, in the Examiner's opinion, it is unclear what is intended by formula VII. The Examiner has asserted that formula VII is described at page 81, line 13 to page 84, line 2, and that indefiniteness arises because variable group  $R_5$ , which is attached to general formula (VII), may itself be a group of general formula (VII). Thus, the Examiner believes the definition of formula VII is circular and has no meaning. In response, it is respectfully submitted that in the instant Amendment, Claim 1 has been amended so that  $R_5$  is a hydrogen atom or a CA group. Thus, in amended Claim 1,  $R_5$  cannot also be general formula (VII).

Furthermore, the Examiner has asserted that Claims 1-8, 10-25, and 29 are indefinite because, in the Examiner's opinion,  $R'$  is defined at page 82, lines 10-15 as representing either a group of formula  $NHR_6R_7$ , for which  $R_6$  and  $R_7$  may independently represent a hydrogen atom or an aliphatic radical, with the further limitation that at least one of  $R_6$  and  $R_7$  may independently represent a hydrogen atom or an aliphatic radical, with the further limitation that at least one of  $R_6$  or  $R_7$  must be different from hydrogen and the other must contain between 10 and 22 carbon atoms. Hence, the Examiner is of the opinion that, on one hand, the Claims state that one or both of  $R_6$  and  $R_7$  may be hydrogen, and on the other hand require that neither  $R_6$  nor  $R_7$  can be hydrogen.

In response, it is noted that although the Examiner has referred to  $R'$  in this aspect of the rejection, the Examiner has described  $R$  in Claim 1. With respect  $R$ , Claim 1 has been amended to state *inter alia*, that  $R$  represents "...a group of formula  $NR_6R_7$  for which  $R_6$  and  $R_7$  represent, independently of each other, a hydrogen atom or an optionally fluorinated, linear or branched, saturated or unsaturated aliphatic radical containing 1 to 22 carbons, with at least one of the two

substituents  $R_6$  or  $R_7$  different from hydrogen and containing between 10 and 22 carbon atoms....” This amendment makes clear that at least one of  $R_6$  or  $R_7$  may be different from hydrogen, which is readily clear and understandable to one of ordinary skill in the art in light of the teachings of the instant Application.

The Examiner has also asserted that Claims 2-10 are indefinite because, although they are drawn to subgeneruses of Claim 1, they fail to begin with a definite article. Thus, the Examiner believes one cannot know which compositions are embraced by Claim 1 are also embraced by these Claims. In response, it is respectfully submitted that Claims 2-7 have been amended to begin with the article “The”, and Claim 10 has been amended to begin with the article “A”. In the instant Amendment, Claim 8 has been canceled, without prejudice. Hence, with respect to Claim 8, this rejection is MOOT.

The Examiner has also asserted that Claim 2 is indefinite because, in the Examiner’s opinion, it is ambiguous due to the use of the conjunction “and” immediately after the phrase “on the one hand”. The Examiner believes the claim should be redrafted to require that “the group  $R_1$  is bonded to either one of  $Z$  or  $V$ , or alternatively is bonded to  $Rep$  via  $Y$ . ”

In response, Claim 2 has been amended in the instant Amendment so that it no longer recites the phrases “on the one hand” and “and on the other hand”. Rather, amended Claim 2 states “The compound according to claim 1, wherein said  $R_1$  group is bonded: (a) to  $Z$  or  $V$ , or (b) to  $Y$  of general formula (III). ”

In addition, the Examiner believes Claim 3 is indefinite because, in the Examiner’s opinion, it is unclear what is and what is not a “member”. In response, Claim 3 has been amended so that it no longer recites the term “members”.

The Examiner has also asserted that Claim 4 is indefinite because the Examiner believes it is unclear whether the limitations regarding  $R_3$ ,  $R_4$ , and  $R_5$  are meant to be applied to each

(NR<sub>4</sub>-(CHR<sub>3</sub>)<sub>t</sub>) group, or only to one of several such groups. In the instant Amendment though, Claim 4 has been amended to be directed towards, *inter alia*, the compound of Claim 1, wherein (a) R<sub>3</sub> is a hydrogen atom or a methyl, wherein R<sub>3</sub> need not be identical in each NR<sub>4</sub>-(CH)<sub>t</sub>R<sub>3</sub> group of general formula (VI) should 2≤t≤8; (b) R<sub>3</sub> is a hydrogen atom and R<sub>4</sub> is a hydrogen atom; or (c) R<sub>4</sub> is a hydrogen atom and R<sub>3</sub> is the group of formula (VII) in which R<sub>5</sub> represents a group CA. It is respectfully submitted that amended Claim 4 is readily clear and understandable to one of ordinary skill in the art. In (a) of Claim 4, R<sub>3</sub> is a hydrogen atom or methyl group, and should t of general formula (VI) be greater than or equal to 2 and less than or equal to 8, then every R<sub>3</sub> must be either a hydrogen atom or methyl group. In (b) of Claim 4, R<sub>3</sub> and R<sub>4</sub> in the compound are hydrogen atoms. Hence, every R<sub>3</sub> and every R<sub>4</sub> in the compound will be hydrogen atoms. In (c) of amended Claim 4, R<sub>4</sub> is a hydrogen atom, R<sub>3</sub> is the group of formula (VII), and R<sub>5</sub> of formula (VII) is a CA group. Thus, amended Claim 4 correctly narrows the scope of Claim 1, upon which it depends, and is readily definite to one of ordinary skill in this art.

The Examiner also believes Claims 6 and 7 are indefinite because they recite “the groups R<sub>6</sub> and R<sub>7</sub>” without proper antecedent basis. In the instant Amendment, Claim 6 has been amended to be drawn to *inter alia*, “The compound according to claim 1, wherein R<sub>6</sub> and R<sub>7</sub> are identical or different, and each represents an optionally fluorinated, linear or branched, saturated or unsaturated aliphatic chain....” Claim 1 discloses R<sub>6</sub> and R<sub>7</sub>. Moreover, amended Claims 6 and 7 correctly narrow scope of Claim 1 by limiting the number of carbons that can be in aliphatic chains R<sub>6</sub> and R<sub>7</sub>. thus, R<sub>6</sub> and R<sub>7</sub> readily have antecedent support in Claim 1, and Claims 6 and 7 are in proper form.

The Examiner further contends that Claim 11 is indefinite because it recites “the analogous lipopolyamines” and “the cyclization” without antecedent bases. It is respectfully

submitted though that in the instant Amendment, Claim 11 has been amended so that it no longer recites the terms “the lipopolyamines” and “cyclization”. Rather, amended Claim 11 is directed towards, *inter alia*, a method for preparing the compound “...comprising the steps of: (a) synthesizing analogous lipopolyamines; and (b) cyclizing the analogous lipopolyamines....” Hence, amended Claim 11 is readily definite to one of ordinary skill in the art.

Furthermore, the Examiner has asserted that Claims 13-23 and 27 are indefinite because they fail to begin with a definite article. Thus, the Examiner believes one cannot know which compositions are embraced by these Claims. In the instant Amendment though, Claims 13 and 21 have been canceled without prejudice, and Claims 14-20, 22-23, and 27 have been amended to begin with definite articles.

The Examiner also believes that Claim 16 is indefinite because, in the Examiner’s opinion, the phrase “such as in particular” recited in the Claim is unclear as to whether the limitations following it are part of the claimed Invention. In the instant Amendment though, Claim 16 has been amended so that it no longer recites the phrase “such as in particular.”

Claims 17 and 18 are also believed to be indefinite for their recitation of the phrase “the nucleic acid” without proper antecedent basis. In response, it is respectfully submitted that Claim 12, upon which Claims 17 and 18 ultimately depend, has been amended to be directed towards, *inter alia*, a composition comprising the compound of claim 1 “...and a nucleic acid....”. Thus, the phrase “the nucleic acid” recited in Claims 17 and 18 has proper antecedent basis.

The Examiner has also asserted that Claim 18 is indefinite because the Examiner believes it is unclear what the metes and bounds of the term “derivatives” are, and it is unclear if the number of peptide units is limited because, in the Examiner’s opinion, the Claim states it is “possible for the number of units to vary between 2 and 10, and then states that the number of

peptide units may be repeated continuously or otherwise". In the instant Amendment though, Claim 18 has been amended such that it no longer recites the term "derivatives". In addition, Claim 18 has been amended to be directed towards, *inter alia*, "The composition according to claim 17, wherein said adjuvant...comprises peptide unit KTPKKAKKP (SEQ ID NO:1) and/or peptide unit ATPAKKAA (SEQ ID NO:2)". Clearly, Claim 18 as amended is readily definite and understandable to one of ordinary skill in the art. Moreover, in its original form, Claim 18 stated that the adjuvant "...consists, *as a whole or in part...*" of peptide units. It is respectfully submitted that the term "comprising" has the same scope as the phrase "consists, as a whole or in part". Hence, the use of the term "comprises" in amended Claim 18 in no way introduces new matter to the Claim.

The Examiner also contends that Claims 24-25 provide for the use of a compound of the instant Invention, but do not set forth any steps involved in the methods/processes. Applicants respectfully remind the Examiner that Claim 25 was deleted in a preliminary amendment filed with the USPTO on 21 November 2000. Moreover, in the instant Amendment, Claim 24 has been canceled, without prejudice.

Finally, Applicants wish to point out to the Examiner that in the instant Amendment, Claim 29 has been canceled, without prejudice. Hence, all the rejections of Claim 29 discussed above are MOOT.

For all of the foregoing reasons, it is respectfully submitted that the Claims as amended herein are readily definite to one of ordinary skill in the art, and that all of these rejections be withdrawn.

### ***The Invention is Enabled***

Claims 24-25 and 28-29 have been rejected under 35 U.S.C. § 112, first paragraph, as failing to comply with the enablement requirement for containing subject matter that was not described in the Specification in such a way as to enable one skilled in the art to which it pertains, or with which it is most nearly connected, to make and/or use the Invention. The Examiner has asserted that Claim 28 is directed towards a method of treating diseases by administering a compound of formula I combined with a nucleic acid capable of correcting said disease. The Examiner has also asserted that Claim 29 is directed towards a method of treating a disease by administering to cells by an intramuscular route a nucleolipid complex comprising a compound of one of Claims 1-9 and a nucleic acid, and that Claims 24-25 are directed to uses of a compound for making a medicament for treating diseases by transfer of nucleic acids into cells.

Applicants respectfully point out to the Examiner that Claim 25 was deleted in a preliminary amendment filed with the USPTO on 21 November 2000. Moreover, in the instant Amendment, Claims 24 and 29 have been canceled, without prejudice. Hence, with respect to Claims 24-25 and 29, this rejection is MOOT.

However, it respectfully submitted that amended Claim 28 as well as new Claims 33-36 are clearly enabled, and should be allowed to issue. In making this the, the Examiner has asserted that at the time the instant Invention was made, successful implementation of gene therapy protocols was not routinely obtainable by those of ordinary skill in the art. In support of the position, the Examiner has cited Verma *et al.* (Nature 389:239-242, 1997) as teaching that “there is still no single outcome that we can point to as a successful story”, and that Verma *et al.* state “Thus far, the problem has been the inability to deliver genes efficiently and to obtain sustained expression”. The Examiner also has asserted that Anderson (Nature 392:25-30 (1998))

confirms the unpredictable state of the art, stating that “there is still no conclusive evidence that a gene-therapy protocol has been successful in the treatment of human disease”, and that “Several major deficiencies still exist including poor delivery systems, both viral and non-viral, and poor gene expression after the genes are delivered.” It is also the Examiner’s position that Romano *et al.* (Stem Cells 18:19-39, 2000) reviewed the general state of gene therapy, and found that the problems relating to gene delivery and expression discussed above persisted, and that Somia and Verma (Nature Review, Genetics 1:91-99, 2000) echoed the beliefs of Romano *et al.*, and noted that delivery vehicles still represented the Achilles heel of gene therapy, and that no single vector existed that had all of the attributes of an ideal gene therapy vector. Hence, it is the Examiner’s opinion that those of the highest skill in the art before, at, and after the time of the instant Invention, felt that the general art of gene therapy was highly unpredictable and could not be practiced with routine success, and that at the time of the instant Invention there was no report of successful gene therapy in humans.

Furthermore, the Examiner has asserted that a review of the prior art and art subsequent to the filing of the instant Application shows that cationic lipids offer poorer efficiency of gene delivery and expression than do the viral vectors that have proved inadequate for the purpose of gene therapy. In support of this position, the Examiner has cited Ross *et al.* (Human Gene Therapy 7:1781-1790, 1996), Table 2 at page 1783, and first full paragraph of column 2 on page 1783; Nishikawa *et al.* (Human Gene Therapy 12:861-870, 2001), which the Examiner believes teaches that factors contributing to the poorer efficiency of cationic nonviral gene delivery include attraction to serum proteins and blood cells, and the difficulty in achieving target-specific gene transfer. The Examiner also has asserted that Nishikawa *et al.* teach that additional problems associated with cationic lipids for delivery include recognition of unmethylated CpG dinucleotides in expression vectors comprising bacterial DNA sequences, release from

endosomes prior to lysosomal degradation, poor stability of DNA in the cytoplasm, and the inhibitory effects of cationic lipids in the nucleus. In further support of his position, the Examiner has also cited Romano (2000) as teaching that cationic lipids as delivery vehicles did not allow specific targeting, had low transfection efficiency, gave only transient expression, were difficult to use *in vivo*, and could give rise to immune responses. Also, the Examiner has cited Miller (Curr. Med. Chem. 10(14):1195-1211, 2003) as teaching that cationic lipids "...are not currently efficient enough to be clinically viable", and Pedroso de Lima *et al.* (Curr. Med. Chem. 10(14):1212-1231, 2003)) as teaching that cationic lipids "...are still far from being viable alternatives to the use of viral vectors in gene therapy"

Furthermore, with respect to gene therapy of muscular dystrophy, the Examiner has asserted that Karpate and Acsadi (Clin. Invest. Med. 17(5):499-509, 1994) teach that several unanswered questions remain to be addressed, such as (a) what type of promoter to use; (b) the method of gene targeting; (c) the required duration of gene expression; and (d) the appropriate route of delivery. Moreover, the Examiner believes that Karpate and Acsadi teach that:

- (i) because of the multinucleate nature of muscle cells, and because dystrophin is deposited near the nucleus where the message is expressed, the majority of myonuclei should acquire a normal allele if most of the muscle fiber is to be covered by dystrophin, but because muscle fibers are surrounded by a well-developed extracellular matrix, efficient gene delivery is problematic;
- (ii) with respect to delivery systems, a variety of techniques, including cationic liposomes, have not produced acceptable results *in vivo*; and

(iii) degradation of the delivered nucleic acid, promoter silencing, mRNA stability or destruction of the transfected cell by host immune response are also problems associated with the expression of delivered genes.

Hence, the Examiner believes that gene therapy with a compound of the present invention to treat muscular dystrophy is unpredictable. Moreover, the Examiner has a similar opinion with respect to gene therapy for treating cystic fibrosis and in antisense treatment.

This rejection is respectfully traversed. Initially, it is respectfully submitted that the Examiner is incorrect in his assertions that gene therapy was an unpredictable technique at the time of the filing of the priority document, and that successful use of gene therapy was not routinely obtainable by those skilled in the art. The filing date for the priority document in this Application is April 2, 1998. Yet, since 1992, approximately 209 U.S. Patents have been issued that contain the phrase “gene therapy” in their title<sup>1</sup>. Indeed, a number of these issued patents were filed with the United States Patent and Trademark Office *prior* to April 2, 1998. Particular examples of such patents include patent number 5,399,346 to Anderson *et al.* entitled *Gene Therapy*<sup>2</sup>, patent number 5,645,829 to Shockley *et al.* entitled *Mesothelial Cell Gene Therapy*<sup>3</sup>, and patent number 5,821,235 to Henning *et al.* entitled *Gene Therapy Using the Intestine*<sup>4</sup>. In issuing these patents, the United States Patent Office has clearly admitted that at the time of filing the priority document in the instant Application, *and even prior thereto*, gene therapy methods were known that were novel, useful, and most importantly, *enabled*, i.e., did not require the performance undue experimentation to perform the therapies. Thus, it is respectfully submitted the Examiner is simply not correct in asserting that gene therapy methods were not predictable at the filing date of the priority document in this matter.

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1 A copy of this list is attached hereto for the Examiner’s review.

2 Filed March 30, 1994 and issued March 21, 1995.

3 Filed June 18, 1993 and issued July 8, 1997.

4 Filed January 20, 1995 and issued October 13, 1998.

Moreover, it is respectfully submitted the Examiner is incorrect in asserting that non-viral vectors are inadequate for the purpose of gene therapy. In support of this assertion, the Examiner is directed to the supplemental Information Disclosure Statement filed with the instant Amendment, which discloses articles that disclose successful exogenous gene expression in mammals using gene therapy non-viral vectors prior to the filing of the priority document for the instant Application, such as:

Wilson *et al.*, J. Biol. Chem. 267(2):963-967 (1992), who teach the use of a DNA-protein complex for *in vivo* delivery;

Alino *et al.*, Biochem. and Biophys. Res. Comm. 204(3):1023-1030 (1994), who teach the use of liposomes for *in vivo* gene therapy;

Canonico *et al.* Am J. Respir. Cell. Mol. Biol. 10:24-29 (1994), which teaches the use of **cationic** liposomes for *in vivo* gene therapy;

Nabel *et al.*, Science. 249:1285-1288 (1990), who teach the use of liposomes for *in vivo* gene therapy;

Zhu *et al.*, Science. 261:209-211 (1993), who teach the use of liposomes for *in vivo* gene therapy; and

Sorscher *et al.*, Human Gene Therapy 5:1259-1277 (1994).

Furthermore, it is respectfully submitted that, contrary to the Examiner's beliefs, the instant Application, as well as knowledge readily available to one of ordinary skill in this art at and prior to the filing of the priority document for the instant Application, provide support for amended Claim 28, as well as new Claims 33-36. Indeed, Examples 11 and 12 teach the successful transfection of cells *in vivo* with a compound of the instant Invention. In each example, DNA encoding luciferase was contacted with a compound of the instant Invention to form a nucleolipid complex, which was then administered to a mouse. Figures 9 and 10 provide

data that clearly shows that after administration of the nucleolipid, luciferase activity was detected in cells *in vivo* that was comparable to the activity seen when naked DNA encoding luciferase was administered. It is respectfully submitted that any DNA sequence, *including those that encode proteins that are useful in treating a disease or disorder* can readily substitute for luciferase using merely routine laboratory techniques, and without the performance of undue experimentation.

Hence, for the foregoing reasons, it is respectfully submitted that amended Claim 28 as well as new Claims 33-36 are clearly enabled, and should be allowed to issue.

### ***The Invention is Novel***

Claims 1-29 have been rejected under 35 U.S.C. § 102(b) as being anticipated by either PCT published patent application WO 99/51581 published October 14, 1999 (the '581 application) or French published application No. 98/04121 published January 19, 1999 (the '121 application). The Examiner has acknowledged these documents are the priority documents listed in the Declaration for the instant Application, and they provide full support for, and thereby anticipate the instant Claims to the extent that the disclosure is enabling. Furthermore, the Examiner has admitted this rejection may be overcome by insertion of a statement into the first line of the instant Specification claiming priority to these documents, provided the priority claim is made during the pending of the Application and within the time limits set forth in the PCT and regulation of the PCT.

As explained above, Claim 25 was deleted in a preliminary amendment filed with the USPTO on 21 November 2000, and in the instant Amendment, Claims 8, 13, 21, 24 and 29 have been canceled, without prejudice. Hence, with respect to these Claims, this rejection is MOOT. Also, for reasons discussed above, the instant Amendment to the Specification to include a

Priority Claim has been made in a timely manner. Thus, it is respectfully submitted that this rejection be withdrawn with respect to all Claims presently pending in the instant Application.

*Fees*

No additional fees are believed to be necessitated by the foregoing Response. However, should this be erroneous, authorization is hereby given to charge Deposit Account No. 18-1982 for any underpayment, or credit any overages.

CONCLUSION

Applicants respectfully request entry of the foregoing amendments and remarks in the file history of the instant Application. The Claims as amended are believed to be in condition for allowance, and reconsideration and withdrawal of all of the outstanding rejections is therefore believed in order. Early and favorable action on the claims is earnestly solicited.

Respectfully submitted,



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TTL/"gene therapy"

PAT.  
NO.

Title

- 1 [RE38,556](#) T Recombinant adeno viruses comprising an inserted gene encoding apolipoprotein and their use in gene therapy for dyslipoproteinemias
- 2 [6,759,394](#) T Cancer gene therapy based on translational control of a suicide gene
- 3 [6,756,201](#) T Diagnostic methods and gene therapy using reagents derived from the human metastasis suppressor gene KAI1
- 4 [6,746,441](#) T Flow-through electroporation system for ex vivo gene therapy
- 5 [6,743,631](#) T Use of human serum resistant vector particles and cell lines for human gene therapy
- 6 [6,743,620](#) T Method for preparing retrovirus vector for gene therapy
- 7 [6,743,444](#) T Method of making microencapsulated DNA for vaccination and gene therapy
- 8 [6,740,331](#) T Apparatus for the delivery of drugs or gene therapy into a patient's vasculature and methods of use
- 9 [6,723,708](#) T Use of lithium (Li<sup>+</sup>) for the preparation of a composition for transfection of a polynucleotide into a cell and compositions useful in gene therapy
- 10 [6,716,824](#) T Treatment of pancreatic adenocarcinoma by cytotoxic gene therapy
- 11 [6,716,622](#) T Tissue-specific self-inactivating gene therapy vector
- 12 [6,709,858](#) T Hyperthermic inducible expression vectors for gene therapy and methods of use thereof
- 13 [6,706,523](#) T Attenuated rabies virus with nucleoprotein mutation at the phosphorylation site for vaccination against rabies and gene therapy in the CNS
- 14 [6,697,669](#) T Skin and muscle-targeted gene therapy by pulsed electrical field
- 15 [6,692,966](#) T Packaging systems for human recombinant adenovirus to be used in gene therapy

16 6,692,737 T In vivo protein production and delivery system for gene therapy  
17 6,689,758 T Gene therapy method  
18 6,689,600 T Formulation of adenovirus for gene therapy  
19 6,677,313 T Method for gene therapy using nucleic acid loaded polymeric microparticles  
20 6,670,188 T Packaging systems for human recombinant adenovirus to be used in gene therapy  
21 6,670,178 T In Vivo production and delivery of insulinotropin for gene therapy  
22 6,669,935 T Delivery of therapeutic agents by gene therapy  
23 6,667,294 T Microencapsulated DNA for vaccination and gene therapy  
24 6,654,636 T Skin and muscle-targeted gene therapy by pulsed electrical field  
25 6,645,942 T Somatic cell gene therapy  
26 6,641,807 T Adenoviral vectors encoding erythropoietin and their use in gene therapy  
27 6,632,670 T AAV vectors for gene therapy  
28 6,630,346 T Gene therapy for obesity  
29 6,627,615 T Methods and compositions for in vivo gene therapy  
30 6,608,038 T Methods and compositions for treatment of diabetes and related conditions via gene therapy  
31 6,602,706 T Packaging systems for human recombinant adenovirus to be used in gene therapy  
32 6,599,698 T Mutated steroid hormone receptors, methods for their use and molecular switch for gene therapy  
33 6,596,515 T Recombinant vector for use in gene therapy for insulin-dependent diabetes mellitus and therapeutic composition thereof  
34 6,592,864 T Cell-based gene therapy  
35 6,589,523 T Agent for gene therapy of dilated cardiomyopathy  
36 6,579,855 T Adenovirus-mediated gene therapy  
37 6,576,463 T Hybrid vectors for gene therapy  
38 6,566,342 T Gene therapy by secretory gland expression  
39 6,562,335 T NK-1 receptor antagonists for prevention of neurogenic inflammation in gene therapy  
40 6,551,588 T Tumor radiosensitization using gene therapy  
41 6,544,948 T DELTA.P62, variants thereof, amino acid sequences coding therefor and their uses in gene therapy for cancer  
42 6,544,771 T Retroviral gene therapy vectors and therapeutic methods based thereon  
43 6,537,813 T Concurrent flow mixing methods and apparatuses for the preparation of gene therapy vectors and compositions prepared thereby  
44 6,537,784 T Self-regulated apoptosis of inflammatory cells by gene therapy  
45 6,537,542 T Targeted introduction of DNA into primary or secondary cells and their use for gene therapy and protein production  
46 6,531,456 T Gene therapy for the treatment of solid tumors using recombinant adeno-associated virus vectors  
47 6,531,124 T In vivo production and delivery of insulinotropin for gene therapy  
48 6,524,572 T Targeting recombinant virus with a bispecific fusion protein ligand in coupling with an antibody to cells for gene therapy  
49 6,524,571 T Methioninase gene therapy for tumor treatment  
50 6,518,412 T Gene therapy approaches to supply apolipoprotein A-I agonists and their use to treat

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TTL/"gene therapy"

PAT. NO.      Title

51 [6,514,947](#) T Formulated nucleic acid compositions and methods of administering the same for gene therapy

52 [6,514,695](#) T Compositions and methods for intraductal gene therapy

53 [6,508,802](#) T Remote sensing gene therapy delivery device and method of administering a therapeutic solution to a heart

54 [6,506,378](#) T Vesicular monoamine transporter gene therapy in Parkinson's disease

55 [6,503,887](#) T Peroral gene therapy of diabetes and obesity

56 [6,500,422](#) T Methods for preventing graft rejection in transplantation and for producing a universal gene therapy host cell using lymphocyte activation (LAG-3)

57 [6,495,527](#) T Complex of DNA and microparticle of defatted lipid-binding protein for gene therapy

58 [6,495,131](#) T Interleukin-3 gene therapy for cancer

59 [6,482,406](#) T Cell-based gene therapy for the pulmonary system

60 [6,475,795](#) T Use of hyaluronan in gene therapy

61 [6,468,793](#) T CFTR genes and proteins for cystic fibrosis gene therapy

62 [6,461,606](#) T Materials and methods for gene therapy

63 [6,451,601](#) T Transiently immortalized cells for use in gene therapy

64 [6,451,593](#) T Design principle for construction of expression constructs for gene therapy

65 [6,447,768](#) T Methods of gene therapy with a DNA sequence encoding NOS

66 6,436,708 T Delivery system for gene therapy to the brain  
67 6,432,676 T Chimeric gene using the gene or cDNA of insulin, specially for the gene therapy of diabetes  
68 6,413,775 T Polyamine analog-activated SSAT gene therapy  
69 6,410,015 T Gene therapy methods using bone marrow-derived cells expressing blood clotting factors  
70 6,410,011 T Gene therapy for restenosis using an adenoviral vector  
71 6,407,178 T Cationic polymers, complexes associating said cationic polymers with therapeutically active substances comprising at least a negative charge, in particular nucleic acids, and their use in gene therapy  
72 6,395,715 T Uteroglobin gene therapy for epithelial cell cancer  
73 6,375,929 T Gene therapy for inhibition of angiogenesis  
74 6,372,500 T Episomal expression cassettes for gene therapy  
75 6,368,275 T Method and apparatus for diagnostic medical information gathering, hyperthermia treatment, or directed gene therapy  
76 6,361,997 T Genetically modified CD34-negative adherently growing stem cells and their use in gene therapy  
77 6,350,444 T Gene therapy for pulmonary edema using adenovirus vectors encoding Na,K-ATPase  
78 6,344,194 T Method for preparing a viral aerosol and its use in gene therapy treatment  
79 6,342,390 T Lipid vesicles containing adeno-associated virus rep protein for transgene integration and gene therapy  
80 6,342,217 T Radiation enhanced gene therapy for tumors expressing a gene for a viral pyrimidine kinase in the presence of a 5'-halogenated pyrimidine  
81 6,339,139 T Receptor-mediated gene transfer system for targeting tumor gene therapy  
82 6,339,065 T Episomal expression vector for human gene therapy  
83 6,335,010 T Gene therapy in coronary angioplasty and bypass  
84 6,335,009 T Vectors and viruses for use in gene therapy  
85 6,331,528 T Method for treatment in gene therapy and use of guanine derivative therefor  
86 6,331,524 T Organ-specific targeting of cationic amphiphile / DNA complexes for gene therapy  
87 6,323,019 T Design of novel highly efficient HIV based packaging systems for gene therapy  
88 6,322,536 T Minimally invasive gene therapy delivery and method  
89 6,310,196 T DNA construct for immunization or gene therapy  
90 6,306,830 T Gene therapy for congestive heart failure  
91 6,306,652 T Packaging systems for human recombinant adenovirus to be used in gene therapy  
92 6,303,379 T Vivo protein production and delivery system for gene therapy  
93 6,297,371 T Process for the preparation of endotoxin-free or endotoxin-depleted nucleic acids and/or oligonucleotides for gene therapy  
94 6,291,423 T Lipid complexes for transferring at least a therapeutically active substance, in particular a polynucleotide into a target cell and use in gene therapy  
95 6,287,557 T Methods of gene therapy using herpes viral vectors expressing GM-CSF  
96 6,284,242 T Method for enhancing myoblast migration and invasion in the context of gene therapy  
97 6,281,010 T Adenovirus gene therapy vehicle and cell line  
98 6,271,211 T Gene therapy for regulating penile smooth muscle tone

99 6,270,795 **T** Method of making microencapsulated DNA for vaccination and gene therapy  
100 6,268,213 **T** Adeno-associated virus vector and cis-acting regulatory and promoter elements  
capable of expressing at least one gene and method of using same for gene therapy

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TTL/"gene therapy"

PAT. NO.      Title

101 [6,265,212](#) T [Packaging systems for human recombinant adenovirus to be used in gene therapy](#)102 [6,261,834](#) T [Vector for gene therapy](#)103 [6,248,720](#) T [Method for gene therapy using nucleic acid loaded polymeric microparticles](#)104 [6,239,117](#) T [Gene therapy for regulating bladder smooth muscle tone](#)105 [6,228,646](#) T [Helper-free, totally defective adenovirus for gene therapy](#)106 [6,225,290](#) T [Systemic gene therapy by intestinal cell transformation](#)107 [6,221,848](#) T [Protection of the esophagus from chemotherapeutic or irradiation damage by gene therapy](#)108 [6,218,180](#) T [Gene therapy for the treatment of solid tumors using recombinant adeno-associated virus vectors](#)109 [6,217,860](#) T [Gene therapy for solid tumors, papillomas and warts](#)110 [6,214,622](#) T [Targeted introduction of DNA into primary or secondary cells and their use for gene therapy](#)111 [6,211,160](#) T [Method for tolerizing a mammalian patient to administration of gene therapy virus vectors](#)112 [6,210,963](#) T [Recombinant cells from the monocyte-macrophage cell line for gene therapy](#)113 [6,207,648](#) T [Methods of using cytochrome P450 reductase for the enhancement of P450-based anti-cancer gene therapy](#)114 [6,204,251](#) T [Ocular gene therapy](#)

115 6,204,060 **T** Viral vectors and line for gene therapy

116 6,204,000 **T** Diagnostic methods and gene therapy using reagents derived from the human metastasis suppressor gene KAI1

117 6,200,799 **T** Somatic gene therapy to suppress secondary cataract formation following eye surgery

118 6,200,304 **T** Transfection system, its preparation and use in somatic gene therapy

119 6,191,257 **T** Natural or recombinant DNA binding proteins as carriers for gene transfer or gene therapy

120 6,190,907 **T** Retroviral vectors for gene therapy

121 6,187,305 **T** Targeted introduction of DNA into primary or secondary cells and their use for gene therapy and protein production

122 6,174,527 **T** Methods and compositions for gene therapy for the treatment of defects in lipoprotein metabolism

123 6,159,467 **T** In vivo suppression of osteosarcoma pulmonary metastasis with intravenous osteocalcin promoter-based toxic gene therapy

124 6,150,338 **T** Gene therapy for alleviating erectile dysfunction

125 6,140,111 **T** Retroviral gene therapy vectors and therapeutic methods based thereon

126 6,140,087 **T** Adenovirus vectors for gene therapy

127 6,135,976 **T** Method, device and kit for performing gene therapy

128 6,129,705 **T** Drug delivery and gene therapy delivery system

129 6,106,826 **T** Replication competent, avirulent Herpes simplex virus as a vector for neural and ocular gene therapy

130 6,100,033 **T** Diagnostic test for prenatal identification of Down's syndrome and mental retardation and gene therapy therefor

131 6,093,699 **T** Method for gene therapy involving suppression of an immune response

132 6,093,567 **T** Gene therapy for cystic fibrosis

133 6,093,392 **T** Methods and compositions for use in gene therapy for treatment of hemophilia

134 6,083,719 **T** Cytidine deaminase cDNA as a positive selectable marker for gene transfer, gene therapy and protein synthesis

135 6,080,728 **T** Carrier: DNA complexes containing DNA encoding anti-angiogenic peptides and their use in gene therapy

136 6,071,890 **T** Organ-specific targeting of cationic amphiphile/DNA complexes for gene therapy

137 6,068,837 **T** Mesothelial cell gene therapy

138 6,066,624 **T** Gene therapy for solid tumors using adenoviral vectors comprising suicide genes and cytokine genes

139 6,063,630 **T** Targeted introduction of DNA into primary or secondary cells and their use for gene therapy

140 6,054,288 **T** In vivo protein production and delivery system for gene therapy

141 6,051,218 **T** Tumor radiosensitization using gene therapy

142 6,048,729 **T** In vivo protein production and delivery system for gene therapy

143 6,048,524 **T** In vivo production and delivery of erythropoietin for gene therapy

144 6,040,295 **T** Formulated nucleic acid compositions and methods of administering the same for gene therapy

145 6,034,137 **T** Cationic lipids for gene therapy

146 6,033,908 **T** Packaging systems for human recombinant adenovirus to be used in gene therapy

147 [6,030,956](#) **T** [Combination gene therapy for human cancers](#)  
148 [6,027,721](#) **T** [Device and method for encapsulated gene therapy](#)  
149 [6,027,488](#) **T** [Flow-through electroporation system for ex vivo gene therapy](#)  
150 [6,017,896](#) **T** [Purine nucleoside phosphorylase gene therapy for human malignancy](#)

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TTL/"gene therapy"

PAT. NO.      Title

151 [6,010,908](#) T [Gene therapy by small fragment homologous replacement](#)

152 [6,001,816](#) T [Gene therapy for leptin deficiency](#)

153 [5,997,509](#) T [Minimally invasive gene therapy delivery device and method](#)

154 [5,994,128](#) T [Packaging systems for human recombinant adenovirus to be used in gene therapy](#)

155 [5,994,127](#) T [In vivo production and delivery of erythropoietin or insulinotropin for gene therapy](#)

156 [5,993,801](#) T [Gene therapy using stromal cells](#)

157 [5,985,846](#) T [Gene therapy for muscular dystrophy](#)

158 [5,965,126](#) T [use of mutant alkyltransferases for gene therapy to protect from toxicity of therapeutic alkylating agents](#)

159 [5,948,675](#) T [Host-vector system which can be used in gene therapy](#)

160 [5,935,934](#) T [Mutated steroid hormone receptors, methods for their use and molecular switch for gene therapy](#)

161 [5,935,568](#) T [Gene therapy for effector cell regulation](#)

162 [5,922,685](#) T [IL-12 gene therapy of tumors](#)

163 [5,912,236](#) T [Broad-spectrum tumor suppressor genes gene products and methods for tumor suppressor gene therapy](#)

164 [5,911,983](#) T [Gene therapy for Gaucher disease using retroviral vectors](#)

165 [5,910,488](#) T [Plasmids suitable for gene therapy](#)

166 [5,885,971](#) T Gene therapy by secretory gland expression

167 [5,882,877](#) T Adenoviral vectors for gene therapy containing deletions in the adenoviral genome

168 [5,874,534](#) T Mutated steroid hormone receptors, methods for their use and molecular switch for gene therapy

169 [5,869,040](#) T Gene therapy methods and compositions

170 [5,866,551](#) T Recombinant adeno viruses comprising an inserted gene encoding apolipoprotein and their use in gene therapy for dyslipoproteinemias

171 [5,856,153](#) T Suicide genes and new associations of pyrimidine nucleobase and nucleoside analogs with new suicide genes for gene therapy of acquired diseases

172 [5,854,019](#) T Cell-specific gene therapy using as promoter novel promoters for tissue inhibitors of metalloproteinase-3

173 [5,849,287](#) T Gene therapy using stromal cells

174 [5,837,531](#) T Recombinant adenoviruses for gene therapy in cancers

175 [5,836,905](#) T Apparatus and methods for gene therapy

176 [5,831,062](#) T Use of the human interferon consensus gene for gene therapy

177 [5,830,880](#) T Gene therapy of tumors with an endothelial cell-specific, cell cycle-dependent active compound

178 [5,827,703](#) T Methods and composition for in vivo gene therapy

179 [5,827,702](#) T Ocular gene therapy

180 [5,824,655](#) T Anti-transforming growth factor-.beta. gene therapy

181 [5,824,544](#) T Adenovirus vectors for gene therapy

182 [5,821,235](#) T Gene therapy using the intestine

183 [5,772,993](#) T Osteocalcin promoter-based toxic gene therapy for the treatment of calcified tumors and tissues

184 [5,770,580](#) T Somatic gene therapy to cells associated with fluid spaces

185 [5,756,283](#) T Method for improved production of recombinant adeno-associated viruses for gene therapy

186 [5,741,486](#) T Safe vectors for gene therapy

187 [5,714,353](#) T Safe vectors for gene therapy

188 [5,707,618](#) T Adenovirus vectors for gene therapy

189 [5,705,151](#) T Gene therapy for T cell regulation

190 [5,702,384](#) T Apparatus for gene therapy

191 [5,693,536](#) T Gene therapy with MCC

192 [5,681,562](#) T Lymphokine gene therapy of cancer

193 [5,670,488](#) T Adenovirus vector for gene therapy

194 [5,665,350](#) T Cell cycle dependent transplantation and ex vivo gene therapy

195 [5,652,224](#) T Methods and compositions for gene therapy for the treatment of defects in lipoprotein metabolism

196 [5,645,829](#) T Mesothelial cell gene therapy

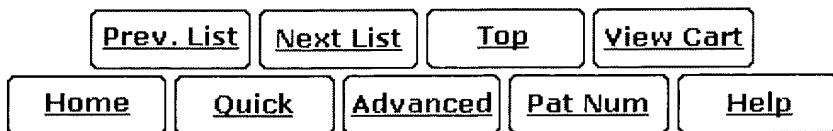
197 [5,637,456](#) T Rapid test for determining the amount of functionally inactive gene in a gene therapy vector preparation

198 [5,631,236](#) T Gene therapy for solid tumors, using a DNA sequence encoding HSV-Tk or VZV-Tk

199 [5,624,820](#) T Episomal expression vector for human gene therapy

200 5,599,712 **T** Protection from ionizing irradiation or chemotherapeutic drug damage by in vivo gene therapy

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TTL/"gene therapy"

PAT.                    Title  
NO.

201 [5,585,362](#) T Adenovirus vectors for gene therapy

202 [5,576,206](#) T Human papilloma virus genes and their use in gene therapy

203 [5,552,311](#) T Purine nucleoside phosphorylase gene therapy for human malignancy

204 [5,496,731](#) T Broad-spectrum tumor suppressor genes, gene products and methods for tumor suppressor gene therapy

205 [5,399,346](#) T Gene therapy

206 [5,252,479](#) T Safe vector for gene therapy

207 [5,240,846](#) T Gene therapy vector for cystic fibrosis

208 [5,219,740](#) T Retroviral gene transfer into diploid fibroblasts for gene therapy

209 [5,166,059](#) T Gene therapy using gene fusions for genetic or acquired disorders

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